# Stem cell model of autism allows testing of new drugs

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Back in May 2009, CIRM held a workshop in which leading scientists discussed ways in which stem cell research could benefit people with autism (the autism workshop report from that meeting is available here). I have two friends with children who are on the spectrum and have seen first-hand the toll the disease takes on the families.

This week, some CIRM grantees published an exciting paper that reflects the hopes of that workshop. The scientists took skin cells from people with a severe form of autism called Rhett syndrome, reverted those cells to an embryonic state, and matured them into neurons. The work was published in the in the November 11 issue of *Cell*. This is the first time scientists have been able to study what amount to autistic neurons in a lab dish.

It turns out they have some abnormalities, as you might expect. According to Technology Review:

"They found that neurons derived from patients with Rett syndrome showed certain abnormalities, including markedly smaller cell bodies, dendrite connections, and decreased cell-to-cell communication.
The best part is that when the team from the Salk Institute and the University of California, San Diego exposed these neurons to a protein called insulin-like growth factor the neurons looked more normal.

This type of work is precisely what the workshop recommended as a starting point. Nerves grown from people with autism are an ideal environment for testing possible therapies and for understanding the disease. The group hopes to test therapeutic options suggested by these findings in mice, and to grow neurons from people with different forms of autism.

At this point the work is far too early to benefit my friends. The scientists still need to better understand the different forms of autism, study this proposed therapy in animals and understand the mechanism better before they can even begin thinking about a human trial. But for a disease that currently has so little clinical hope, even early stage work is a step in the right direction.

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